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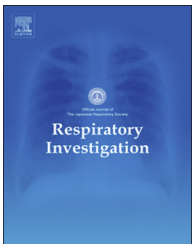

Editorial

Searching Hidden Truth behind Clinical Trials

Global clinical trials that previously focused on common diseases are now designed to study rare lung diseases [1]. Although study of rare lung disease is of significant clinical value, various limitations, such as differences in diagnostic modalities and insurance systems, are yet to be overcome. In cross-cultural studies, efficacy should be objectively evaluated as the primary endpoint, rather than subjective measures, which are reported by the patient, such as quality of life (QOL). A central review of the assessment is normally performed for every clinical trial in order to ensure accurate diagnosis. With regard to the risk–benefit ratio, a periodical assessment of the risk–benefit balance is required to ensure the safety of enrolled patients during the trial, which may be the sponsor's responsibility.

Pharmaceutical companies normally design clinical trial protocols, which has a positive outcome. Therefore, these companies need to acquire a mandatory IPF sub-population for a candidate agent.

This sub-population may be different from IPF patients covered glorious diversity in the real world. Large-scale trials with interferon gamma failed to produce significant results [2]. The candidate agent is expected to benefit a larger population of IPF patients; however, the sub-population was only benefitted at predefined endpoints. These results revealed that statistically significant risks and benefits were experienced by the sub-population of IPF patients. The benefits and risks during treatment with anti-fibrotic agents have been recently studied in certain IPF phenotypes.

The results of a severity grading method in Japan revealed that the clinical features of IPF vary during disease progression [3]. A majority of clinical trials have enrolled mild to moderate IPF patients in order to obtain a safe positive outcome with regard to the primary endpoint. How do we treat the remaining IPF patients using this candidate agent? In other words, how do we obtain proof of efficacy of these trials for other IPF patients outside this study? To address these questions, observational analysis is essential even in a trial-directed proof of concept (POC) [4].

Although translational research based on basic research has a scientific basis, observational research is essential for live clinical practice. Instead of conducting studies on single molecular inhibition, reverse-translational research based on a clinical observational process, which leads to regulatory science, along with poly-pharmacological logistics is required [5].

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